



**RESEARCH *in* YOUR
BACKYARD**

Developing Cures, Creating Jobs

Pharmaceutical clinical trials in
**EASTERN
NORTH CAROLINA**

Executive



Summary

Clinical trials in EASTERN NORTH CAROLINA

This report shows how biopharmaceutical research companies continue to be vitally important to the economy and patient health in Eastern North Carolina.

Since 2004, biopharmaceutical research companies are conducting or have conducted more than 700 clinical trials of new medicines

in Eastern North Carolina in collaboration with clinical research centers and hospitals. These clinical trials have investigated or are investigating some of Eastern North Carolina's biggest health care challenges, including rheumatoid arthritis, cancer, diabetes, cardiovascular disease and kidney disease.

CLINICAL TRIALS IN EASTERN NORTH CAROLINA ARE A VITAL PART OF THE FDA DRUG APPROVAL PROCESS

In the development of new medicines, clinical trials are conducted to prove therapeutic safety and effectiveness and compile the evidence needed for the U.S. Food and Drug Administration (FDA) to approve new treatments.

Clinical tests of new drugs are conducted in three phases and, on average, account for nearly seven of the more than 10 years it takes to bring a new drug from development to patients. Clinical

trials are responsible for more than half of the \$2.6 billion average cost of developing one new innovative medicine.

All clinical trials must be reviewed and approved by an Institutional Review Board (IRB) in advance; an independent committee of physicians, statisticians, local community advocates and others to ensure a trial is ethically conducted and patient rights are protected.

Clinical Trials in Eastern North Carolina since 2004—Completed and Open

All Clinical Trials	Open Clinical Trials
715	120

Source: www.clinicaltrials.gov. Search criteria: Cities and Towns of Eastern North Carolina, United States; Phase early 1, 1, 2, 3; Industry only, first received on or after 1/1/2004. Search performed 9/19/2017. Open clinical trials are recruiting, not yet recruiting, or expanded access.

Executive Summary (cont.)

"East Carolina University is honored that globally recognized industry sponsors are bringing clinical trials to our patients, as through these opportunities we are contributing to the advancement of medicine and more importantly providing cutting-edge therapies to our patients."

Devon Kuehn, MD,
Director of Pediatric Research
East Carolina University

"Cancer knowledge and treatment is constantly evolving and the most up to date therapies are drugs and interventions administered under research protocols. Children treated on research protocols generally have better outcomes than those treated off research protocols. As a pediatric oncologist at East Carolina University, I am proud to be a part of Children's Oncology Group, a network of providers and centers that conduct research and clinical trials to ensure our patients are provided with the most advanced treatment options. This way we can improve outcomes and decrease the suffering of our children with cancer and provide their families with hope."

Beng Fuh, MD,
Pediatric Oncologist
East Carolina University

CLINICAL TRIALS OFFER IMPORTANT THERAPEUTIC OPTIONS FOR PATIENTS

For patients, clinical trials offer the potential for another therapeutic option. Clinical tests may provide a new avenue of care for some chronic disease sufferers who are still searching for the medicines that are best for them.

Some clinical trials are conducted to compare existing treatments and some are done to explore whether a drug is appropriate for a different patient population, such as children or the elderly. Still others are conducted to find ways to make existing approved drugs more effective and easier to use with fewer side effects.

ECONOMIC IMPACT OF THE BIOPHARMACEUTICAL SECTOR IN NORTH CAROLINA

Biopharmaceutical research companies have been and continue to be a good source of jobs, tax revenue and research spending in North Carolina.

A study by TEconomy Partners found that in 2014, the industry supported more than 259,000 jobs throughout North Carolina. Wages and benefits for employees whose jobs were supported by the biopharmaceutical sector resulted in more than \$2.7 billion in federal taxation and \$407.2 million in state taxes.

Biopharmaceutical research companies supported the generation of \$77.6 billion in economic activity in the state, including the direct economic output of the sector itself, the output of the sector's vendors and suppliers and the output generated by the buying power of its workforce.

Company employees in North Carolina include life science researchers, management executives, office and administrative support workers, production workers, engineers, architects, computer and math experts, and sales representatives. Biopharmaceutical companies also supported the jobs of their vendors and suppliers, including construction and IT firms. And the employees of biopharmaceutical companies help to support local restaurants, day care centers and other community businesses.

ECONOMIC IMPACT OF CLINICAL TRIALS IN NORTH CAROLINA

A separate study by Battelle Technology Partnership Practice found that in 2013 alone, there were 1,779 active industry-sponsored, site-based clinical trials in North Carolina, with an estimated enrollment of 45,524 North Carolina residents. Oncology had the leading clinical trial enrollment in the state.

The investment of these site-based clinical trials was more than \$400 million and the estimated total economic impact was more than \$1 billion.

“North Carolina is a global life science leader with more than 650 companies employing more than 63,000 people in high-paying jobs across the sector. It generates \$86 billion in economic activity and \$2.2 billion in state and local taxes. Most of that involves some form of health care, ranging from diagnostics and medical devices to pharmaceutical manufacturing like that from Greenville’s Patheon, now becoming Thermo Fisher, as well as Mayne Pharma. In fact, Eastern North Carolina, led by East Carolina University, is playing an ever-increasing role in the R&D and testing needed to bring these new products to market. NCBiotech is committed to leading this transformational endeavor.”

**Mark Phillips, NCBiotech
Vice President of Statewide
Operations and Executive Director
of the Eastern Office in Greenville**

Open Clinical Trials in Eastern North Carolina by Disease	
Disease	Number of Trials
Arthritis/Musculoskeletal Disorders	7
Autoimmune Diseases	5
Blood Disorders	9
Cancer	35
Cardiovascular Diseases	6
Diabetes	7
Eye Disorders	2
Gastrointestinal/Esophageal Diseases	23
Infectious Diseases	7
Kidney Diseases	6
Liver Diseases	3
Respiratory Diseases	3
Other Diseases	7
Total	120

Source: www.clinicaltrials.gov. Search criteria: Cities and Towns of Eastern North Carolina, United States; Phase early 1, 1, 2, 3; Industry only; first received on or after 1/1/2004. Search performed 9/19/2017. Open clinical trials are recruiting, not yet recruiting, or are expanded access.

Patient Resources & Directory

WHAT IS THE CLINICAL TRIAL EXPERIENCE?

Clinical trials are research studies that generate data to support FDA approval of a new medicine or a new indication for an existing medication. They also grant participants early access to new medicines, which are being developed to help combat chronic and serious diseases. By volunteering for a clinical trial, patients take an active role in their health care by helping researchers test new treatments. In Eastern North Carolina, 715 clinical trials since 2004 have targeted diseases and conditions like rheumatoid arthritis, cancer, diabetes, cardiovascular disease and kidney disease.

PHASES OF CLINICAL TRIALS

There are three phases of clinical testing used to evaluate potential new medicines:

PHASE I—Researchers test the drug in a small group of people, usually between 20 and 100 healthy adult volunteers, to evaluate its initial safety and tolerability profile, determine a safe dosage range and identify potential side effects.

PHASE II—The drug is given to volunteer patients, usually between 100 and 500 people, to study its efficacy, identify an optimal dose and to further evaluate its short-term safety.

PHASE III—The drug is provided to a larger, more diverse patient population, often involving between 1,000 and 5,000 patients (but sometimes many more thousands), to generate statistically significant evidence to confirm its safety and effectiveness. They are the longest studies and usually take place in multiple sites around the world.

LEARNING ABOUT AND ACCESSING CLINICAL TRIALS

Patients can learn about clinical trials in several ways. Health care providers are aware of clinical trials being conducted at hospitals, universities and other leading health care facilities, and these institutions can be valuable sources of information for patients looking to participate. Patients can also use hospital and university websites to find the trials being conducted in their area. Information on clinical trials at East Carolina University can be found at www.ecu.edu/clinicaltrials/.

More information about clinical trials in Eastern North Carolina and how to volunteer for one can be found at www.centerwatch.com, a PhRMA-recommended website.

WHAT TO EXPECT

Since clinical trials are often conducted in a doctor's office, patients may need to devote more time to physician visits and physical examinations. They may also have additional responsibilities, like keeping a daily log of their health. All prospective participants must sign an informed consent document saying they understand that the clinical trial is research, and that they can leave the trial at any time. After consulting with their health care providers, patients can volunteer to participate, leading to a pre-screening interview. If they fit the criteria and requirements of the test, they can be enrolled.

PATIENT EXPENSES

Patients should ask during pre-screening interviews what it will cost them to participate in a clinical trial. Clinical trial sponsors usually pay for all research-related expenses and additional testing or physician visits required by the trial. Patients or their insurance companies may be asked to pay for any routine treatments of their disease. And it's important to know some health plans do not pay for clinical trials.

Patients should make it a point to learn if they or their insurance company will be assessed any fees and should determine if their insurance company will cover the expense of routine examinations. Patients who live a distance from the trial site should learn the clinic's policy for covering travel costs and living expenses.

The National Cancer Institute, for example, makes patients responsible for their own travel costs for the initial screening visits. Once a patient is enrolled, the Institute will pay for transportation costs for all subsequent trial-related visits. These patients will receive a small per diem for food and lodging.

EXPANDED ACCESS

Successful completion of the clinical trials is required to demonstrate to the FDA that an investigational drug is safe and effective, so that it can be approved and made available to a broad patient population. Clinical trials are the primary route by which patients can participate in the drug development process, receive access to unapproved investigational drugs and contribute to the collection of safety and efficacy data necessary for FDA approval.

For patients with a serious or life-threatening disease who are ineligible or unable to participate in a clinical trial, use of an unapproved investigational drug through an expanded access program may be an option. The current FDA process for a patient to gain access to an investigational drug through expanded access was established in 2009 in close consultation with patients, physicians and the biopharmaceutical industry. Expanded access programs are part of many biopharmaceutical companies' commitment to patients.

*For more information about **the drug development and approval process in the United States**, see page 12*

LOCAL PATIENT ADVOCACY GROUPS

Patient advocacy groups in Eastern North Carolina provide an exceptional resource for patients to connect and learn more about their condition and what treatment options are available in the state. These groups also provide an important voice on behalf of patients to protect their access to medicine and treatment.

The following are just a few major groups that work on behalf of patients in Eastern North Carolina and may provide more information to patients with further questions.

Alzheimer's Association

EASTERN NORTH CAROLINA CHAPTER
The Cumberland Building
3739 National Drive, Suite 110
Raleigh, NC 27612
(919) 803-8285

American Cancer Society

McCONNELL-RAAB HOPE LODGE
930-A Wellness Drive
Greenville, NC 27834
(252) 695-6143

American Cancer Society

GREENVILLE OFFICE
930 Wellness Drive, Suite B
Greenville, NC 27834
(252) 695-9028

American Diabetes Association

RALEIGH OFFICE
2418 Blue Ridge Road, Suite 206
Raleigh, NC 27607
(919) 743-5400

American Heart Association

EASTERN NORTH CAROLINA OFFICE
3131 RDU Center Drive, Suite 100
Morrisville, NC 27560
(919) 463-8300

American Lung Association

RALEIGH CHAPTER
514 Daniels Street, Suite 109
Raleigh, NC 27605
(919) 792-1641

Arthritis Foundation

NORTH CAROLINA OFFICE
4530 Park Road, Suite 230
Charlotte, NC 28209
(704) 705-1808

Epilepsy Foundation of North Carolina

PIEDMONT ONE
1920 W. First Street, Suite 5541 A
Winston-Salem, NC 27104
(336) 716-2320

NAMI North Carolina

NATIONAL ALLIANCE ON MENTAL ILLNESS
309 W. Millbrook Road, Suite 121
Raleigh, NC 27609
(919) 788-0801

OTHER PATIENT RESOURCES

PARTNERSHIP FOR PRESCRIPTION ASSISTANCE (PPA):

The Partnership for Prescription Assistance has helped more than 336,000 North Carolina patients access free or nearly free prescription medicines for residents who are underinsured or uninsured within the state. Patients should go to www.pparx.org for more information. The on-line process takes about 15 minutes, and you'll find out instantly if you're likely to be eligible for help.

HEALTHCARE READY: Healthcare Ready is a tool activated to help keep emergency responders informed on the status of the biopharmaceutical supply chain in the event of a natural disaster or emergency. Healthcare Ready's Rx Open tool was deployed in 11 states and the District of Columbia, and helped victims and evacuees who needed to fill or re-fill their prescriptions find open pharmacies. Healthcare Ready also helped emergency responders with critical information on the challenges facing supply chain partners relating to electricity, fuel and transportation issues. See more at www.healthcareready.org.

Clinical Trial Policy Resources

THE BIOPHARMACEUTICAL SECTOR'S ROLE IN THE ECONOMY

America's biopharmaceutical research companies serve as the foundation for one of the country's most dynamic innovation and business ecosystems. The biopharmaceutical industry is among the most research and development (R&D) intensive industries in the United States. In fact, the sector accounts for the single largest share of all U.S. business R&D, accounting for approximately 17 percent of all R&D spending by U.S. businesses. The industry and its large-scale research and manufacturing supply chain supports high-quality jobs across the U.S. economy.

Biopharmaceutical companies invest 12 times more in R&D per employee than manufacturing industries overall.

The biopharmaceutical industry supported more than 4.4 million jobs across the U.S. economy in 2014, according to a study by TEconomy Partners.

Since 2000, biopharmaceutical companies that are members of the Pharmaceutical Research and Manufacturers of America have invested more than \$600 billion in R&D in the search for new treatments and cures.

ECONOMIC IMPACT OF THE BIOPHARMACEUTICAL SECTOR IN NORTH CAROLINA

Biopharmaceutical research companies have been and continue to be a source of quality jobs, tax revenue and research spending in North Carolina. A TEconomy Partners study found that the biopharmaceutical sector:

- Supported more than 259,000 jobs throughout North Carolina in 2014.
- Supported the generation of \$7.6 billion in economic activity in the state.
- Resulted in more than \$2.7 billion in federal taxation and \$407.2 million in state taxes through jobs supported by the biopharmaceutical sector.

For more information on the **economic impact of the biopharmaceutical industry in North Carolina**, see page 2.

PUBLIC-PRIVATE PARTNERSHIPS AND LOCAL COLLABORATION

The following are just a few of the prominent institutions in Eastern North Carolina that biopharmaceutical research companies are collaborating with on clinical trials for new medicines.

- **21st Century Oncology**, Greenville
- **AG Clinical Research**, Greenville
- **Albemarle Clinical Trials**, Elizabeth City
- **Albemarle Medical Center**, Elizabeth City
- **Allergy Partners of East Carolina**, Greenville
- **Atlantic Medical Group**, Kinston
- **Boice Willis Clinic**, Rocky Mount
- **Brody School of Medicine, East Carolina University**, Greenville
- **Cancer Center of the Carolinas**, Greenville
- **Carolina Digestive Diseases**, Greenville
- **Carolina East Family Practice**, Greenville
- **Carolina Research Center**, Greenville
- **Coastal Carolina Health Care**, New Bern
- **CTMG**, Greenville
- **Diabetes Research Center at East Carolina University**, Greenville
- **East Carolina Heart Institute at East Carolina University**, Greenville
- **East Carolina University School of Dental Medicine**, Greenville
- **Eastern Carolina Foot & Ankle Specialists**, Greenville
- **Eastern Carolina Medical Clinic**, Benson
- **Eastern Carolina Physicians**, Kinston
- **Eastern Carolina Women's Center**, New Bern
- **Eastern Institute of Medical Sciences**, Greenville
- **Eastern Nephrology Associates**, Greenville
- **Eastern North Carolina Medical Group**, Rocky Mount
- **Eastern Urological Associates**, Greenville
- **Endocrine Research Physicians**, Greenville
- **ESI Medical Research**, Kinston
- **Farmville Internal Medicine**, Farmville
- **Forsyth Regional Cancer Center**, Winston-Salem
- **GHS (Greenville Health System) Cancer Institute**, Greenville
- **Gynecologic Oncology Network**, Greenville
- **Institute for Translational Oncology Research**, Greenville
- **Johnston Memorial Hospital**, Smithfield
- **Johnston Therapeutic Wound Clinic**, Smithfield
- **Kinston Medical Specialists**, Kinston
- **Leo W. Jenkins Cancer Center at ECU Medical School**, Greenville
- **Marion L. Shepard Cancer Center at Beaufort County Hospital**, Washington
- **New Bern Cancer Care**, New Bern
- **North Carolina Heart and Vascular**, Clayton, Smithfield
- **Orthopedics East**, Greenville
- **Physicians East**, Farmville, Greenville, Winterville
- **Pitt County Memorial Hospital**, Greenville
- **PMG Research of Rocky Mount**, Rocky Mount
- **South Oncology Research**, Grifton
- **Southeast Cancer Control Consortium**, Goldsboro
- **Southeastern Medical Oncology Center**, Goldsboro
- **Southeastern Medical Oncology Clinic**, Wilson
- **Southern Gastroenterology Associates**, New Bern
- **Three Rivers Health and Rehabilitation**, Windsor
- **Vidant Medical Center**, Greenville
- **Virginia Oncology Associates**, Elizabeth City
- **Wayne Memorial Hospital**, Goldsboro
- **Wilson Medical Center**, Wilson

Collaborations between the biopharmaceutical research industry and universities play an important role in the development of new medicines. In the United States, there are more than 7,100 open clinical trials¹ being sponsored by the biopharmaceutical industry, universities, individuals and organizations combined. These trials represent studies being funded by industry, research collaboration studies and research the other groups are undertaking on their own.

In **Eastern North Carolina**, of the 120 open clinical trials involving the biopharmaceutical research industry, East Carolina University is collaborating on more than 21 clinical trials.

THE STATE OF DISEASE IN NORTH CAROLINA

More than 10 million people live in North Carolina¹, and many are dealing with disease and disability from asthma to cancer and from diabetes to heart disease.

Selected Disease Statistics in North Carolina	
Disease	Health Statistic
Alzheimer's Deaths, 2015 ²	3,803
Asthma Prevalence-Adults, 2015 ³	8.2 percent
Cancer New Cases, 2017 ⁴	56,900
Cancer Deaths, 2017 ⁴	20,020
Chronic Lower Respiratory Diseases, 2015 ²	5,221
Diabetes Prevalence-Adults, 2015 ³	10.7 percent
Diabetes Deaths, 2015 ²	2,743
Heart Disease Deaths, 2015 ²	18,467
HIV-Number Living with a Diagnosis, 2015 ³	28,897
Mental Illness-Adults, 2015 ³	1,429,000
Influenza / Pneumonia Deaths, 2015 ²	2,113
Stroke Deaths, 2015 ²	5,028

Source: 1. U.S. Census Bureau 2. North Carolina Center for Health Statistics 3. Kaiser Family Foundation, State Health Facts 4. American Cancer Society

¹ Data collected from www.clinicaltrials.gov. Search criteria: United States, Phase 0, 1, 2, 3; Industry and Other, first received on or after 1/1/2004. Search performed 10/2/2017. Open clinical trials are recruiting, not yet recruiting, or are expanded access.

NORTH CAROLINA CLINICAL TRIALS AND SPECIAL POPULATIONS: CHILDREN, OLDER AMERICANS AND WOMEN

- Children under the age of 18 make up nearly 23 percent of the population in North Carolina. Pediatric clinical trials are being conducted in the state for solid tumors, leukemia, diabetes, Duchenne muscular dystrophy, epilepsy, cystic fibrosis and hemophilia A, among others.
- North Carolinians aged 65 and older account for 15.1 percent of the states' population. In North Carolina, clinical trials are recruiting older people to study potential treatments for diseases such as Alzheimer's disease, chronic obstructive pulmonary disease, lung cancer, heart failure, macular degeneration, epilepsy and osteoarthritis.
- Women and girls make up 51.3 percent of the population in North Carolina. Clinical trials are recruiting women for studies on medicines for cervical cancer, ovarian cancer, vaginal infections and postpartum depression, among others.

Open Clinical Trials in Eastern North Carolina for Special Populations	
Population	Number of Trials
Children (birth-17)	17
Seniors (66 and older)	107
Women (only)	7

Source: www.clinicaltrials.gov. Search criteria: Cities and Towns of Eastern North Carolina, United States; Phase early 1, 1, 2, 3; Industry only; first received on or after 1/1/2007. Search performed 9/19/2017. Open clinical trials are recruiting, not yet recruiting, or expanded access.

SCIENCE AND CLINICAL TRIALS

Some of the medicines in clinical testing in Eastern North Carolina feature revolutionary medical technologies. For example:

- A monoclonal antibody for the treatment of idiopathic pulmonary fibrosis was studied in a clinical trial in **Greenville**.
- A second-generation medicine for leukemia that blocks the activation of a receptor which is mutated in about one-third of all patients with acute myeloid leukemia is being tested in patients at the Leo W. Jenkins Cancer Center at East Carolina University in **Greenville** and at Kinston Medical Specialists in **Kinston**.
- A medicine that targets a mutation in the gene that encodes BRAF kinase is being studied to treat melanoma at locations in **Goldsboro, Kinston** and **Wilson**.
- A medicine in development for the treatment of HIV infection prevents the entry of HIV into the cells and prevents it from infecting immune cells while preserving normal immune function. The medicine is being studied in clinical trials at East Carolina University in **Greenville**.

The innovative treatments that are being developed today are helping to expand the frontiers of science and could lead to more and better treatments for patients in the future. In Eastern North Carolina, this innovation is the result of a successful collaboration between biopharmaceutical companies and local research institutions.

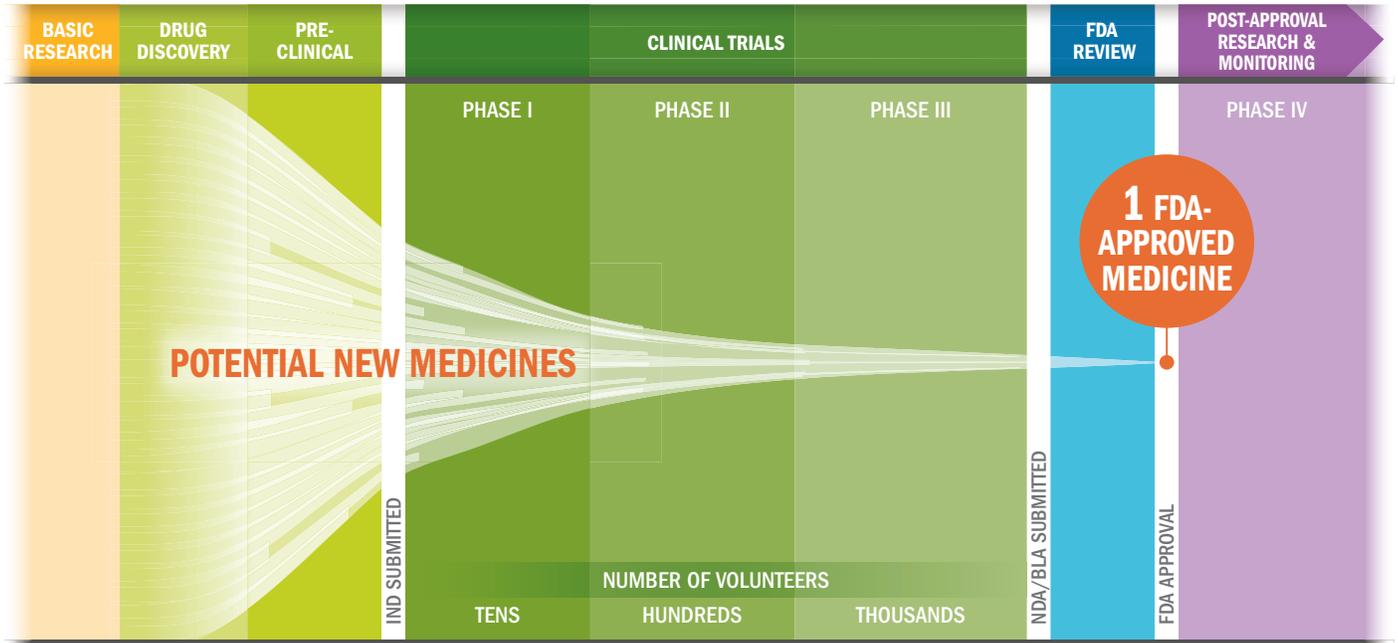
Clinical Trials in Eastern North Carolina by Location

Location	All Clinical Trials	Open Clinical Trials
Benson	3	1
Clayton	4	—
Cleveland	1	—
Columbia	1	—
Elizabeth City	48	2
Farmville	4	1
Goldsboro	62	12
Greenville	393	57
Grifton	1	—
Kinston	65	27
Nashville	1	—
New Bern	128	18
Rocky Mount	58	17
Washington	23	2
Wilson	27	9
Windsor	2	—
Winton	1	—

Source: www.clinicaltrials.gov. Search criteria: Cities and Towns of Eastern North Carolina, United States; Phase early 1, 1, 2, 3; Industry only; first received on or after 1/1/2004. Search performed 9/19/2017. Open clinical trials are recruiting, not yet recruiting, or are expanded access.

THE BIOPHARMACEUTICAL RESEARCH AND DEVELOPMENT PROCESS

From drug discovery through FDA approval, developing a new medicine takes at least 10 years on average and costs an average of \$2.6 billion.* Less than 12% of the candidate medicines that make it into Phase I clinical trials will be approved by the FDA.



Key: IND: Investigational New Drug Application, NDA: New Drug Application, BLA: Biologics License Application

* The average R&D cost required to bring a new, FDA-approved medicine to patients is estimated to be \$2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.

Source: PhRMA adaptation based on Tufts Center for the Study of Drug Development (CSDD) Briefing: "Cost of Developing a New Drug," Nov. 2014. Tufts CSDD & School of Medicine and US FDA Infographic, "Drug Approval Process," <http://www.fda.gov/downloads/Drugs/ResourcesForYou/Consumers/UCM284393.pdf> (accessed Jan. 20, 2015).

